

Title (en)

ADENO-ASSOCIATED VIRUS VARIANT CAPSIDS AND USE FOR INHIBITING ANGIOGENESIS

Title (de)

KAPSID MIT ADENO-ASSOZIIERTEN VIRENVARIANTEN UND VERWENDUNG ZUR HEMMUNG DER ANGIOGENESE

Title (fr)

CAPSIDES VARIANTES DE VIRUS ADÉNO-ASSOCIÉS ET LEUR UTILISATION POUR INHIBER L'ANGIOGENÈSE

Publication

EP 4272728 A2 20231108 (EN)

Application

EP 23186524 A 20181126

Priority

- US 201762590976 P 20171127
- US 201862664726 P 20180430
- EP 23152950 A 20181126
- EP 18881166 A 20181126
- US 2018062478 W 20181126

Abstract (en)

Provided herein are variant adeno-associated virus (AAV) capsid proteins having one or more modifications in amino acid sequence relative to a parental AAV capsid protein, which, when present in an AAV virion, confer increased infectivity of one or more types of retinal cells as compared to the infectivity of the retinal cells by an AAV virion comprising the unmodified parental AAV capsid protein. Also provided are recombinant AAV virions and pharmaceutical compositions thereof comprising a variant AAV capsid protein as described herein, methods of making these rAAV capsid proteins and virions, and methods for using these rAAV capsid proteins and virions in research and in clinical practice, for example in, e.g., the delivery of nucleic acid sequences to one or more cells of the retina for the treatment of retinal disorders and diseases.

IPC 8 full level

A61K 9/00 (2006.01)

CPC (source: EP IL KR US)

A61K 9/0048 (2013.01 - IL US); **A61K 48/0008** (2013.01 - EP IL KR US); **A61K 48/005** (2013.01 - EP IL KR US);
A61K 48/0075 (2013.01 - IL US); **C07K 14/005** (2013.01 - EP IL KR US); **C07K 16/22** (2013.01 - EP IL KR US); **C12N 7/00** (2013.01 - US);
C12N 15/86 (2013.01 - EP IL KR); **A61K 9/0048** (2013.01 - EP); **A61K 48/0075** (2013.01 - EP); **C12N 2750/14122** (2013.01 - EP IL KR US);
C12N 2750/14143 (2013.01 - EP IL KR US); **C12N 2750/14145** (2013.01 - EP IL KR)

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DOCDB simple family (publication)

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DOCDB simple family (application)

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